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Platinum Priority – Review – Voiding Dysfunction

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A Narrative Review of Patient-reported Outcomes in Overactive Bladder: What is the Way of the Future?

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Abstract

Context: Debate exists on overactive bladder (OAB) treatment-response assessment in clinical trials and the nature and shortcomings of the different endpoints used in OAB clinical research.

Objective: To evaluate current evidence and tools that measure OAB treatment response in clinical trials and to inform the development of a new multidimensional patient-reported outcome (PRO) that could be used as a primary endpoint in OAB trials.

Evidence acquisition: We conducted a narrative review of OAB literature available in the PubMed database published between January 1, 2004 and June 30, 2015. Eighty articles were selected for full text review.

Evidence synthesis: The assessment of treatment outcomes in OAB is challenging due to the heterogeneity of symptoms and reliance on PROs. OAB studies report a high level of placebo effect and the placebo response is poorly understood. We found significant correlations between PRO measures and bladder diaries. There is evidence of several issues with the bladder diary: burden, over/underestimation, recall period, and lack of validation. Trials for other conditions—interstitial cystitis, benign prostatic hyperplasia, headache, and restless legs syndrome—have used symptom scales rather than diaries to measure treatment outcomes and some now incorporate PRO measures as primary, coprimary, and secondary endpoints. The International Consultation on Incontinence Research Society recommends evaluation of satisfaction, symptoms, health-related quality of life, and adverse events.

Conclusions: There is strong evidence of the shortcomings in current approaches to measuring OAB outcomes in clinical trials and recognition that a new simpler approach which incorporates symptom and health-related quality of life assessment could provide a more comprehensive, standardized approach to OAB assessment.

Patient summary: Overactive bladder is a urinary syndrome. Individuals experience different symptoms to varying degrees, which poses difficulties in accurately measuring the effect of treatment. This review found evidence and recommendations that propose a simpler but more comprehensive way to measure treatment outcomes.

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1. Introduction

The International Continence Society defines overactive bladder (OAB) symptom complex as “urinary urgency, usually accompanied by frequency and nocturia, with or without urgency urinary incontinence (UUI), in the absence of urinary tract infection or other obvious pathology” [1]. This symptom-based definition is a useful starting point in terms of diagnosing patients; however, in terms of evaluating the impact of interventions, it fails to address what is most important to patients. Patients seek treatment because their symptoms affect their health-related quality of life (HRQoL) [2]. Given the heterogeneity of symptoms and multifaceted impact of OAB, measurement of outcomes in clinical trials is complicated, and researchers are confronted with the problem of balancing basic assessment with obtaining a comprehensive picture of patient outcomes [3]. Goldman et al [4] highlighted the lack of formal guidance and the significant heterogeneity of both response and nonresponse definitions in a systematic review of OAB treatment endpoints. Goldman et al [4] reports on the heterogeneity of symptom-based and patient-reported outcome measures (PROMs)-based definitions of treatment response/nonresponse. For example, while most studies defined UUI treatment response as a 50–100% reduction in UUI episodes [4], others included a reduction of ≥ 2 episodes/wk [5], $\geq 50\%$ reduction in incontinence pad weight [6], an increase in ≥ 1 continent d/wk [5], or 3–7 consecutive dry d [7]. The symptoms of urgency and frequency have also been used as endpoints with similar heterogeneity in the criteria used for definitions of success.

As evidenced by the above discussion, by recording frequency, volume, and number of incontinence episodes the bladder diary is at the core of every OAB assessment and represents the *gold standard* investigation [8]. Additional information may include the number of pads used and quantity of fluid intake [9]. The diary is clearly a useful tool not only in the initial patient evaluation as it allows clinicians to appropriately diagnose and plan an intervention, but also in objectively defining response to therapy.

See Figure 1 for an overview of recommended endpoints in OAB.

To capture the impact of symptoms on patients, several psychometrically-validated PROMs exist [10]. These include the Overactive Bladder Symptom Score (OABSS) [11], the Overactive Bladder Questionnaire (OAB-q) [12], the King's Health Questionnaire [13], and the Patient Perception of Bladder Condition [14]. PROMs are routinely included as secondary endpoints in trials alongside diaries [15]. While some trials rely solely on primary nonbladder diary-based PROMs endpoints [16], other frequently used PROMs include global assessments, satisfaction, and goal attainment scaling [17].

To understand, support, and inform the development of a new multidimensional PROMs that could be used to replace bladder diaries as a primary or key secondary endpoint in clinical trials, we conducted a review of literature published within the past 10 yr on OAB treatment-response assessments. In particular, addressing the key issues of: (1) whether the definition of treatment response/nonresponse should include a symptom assessment, (2) should PROMs provide information about whether a reduction in symptoms actually improves patients' lives, and (3) use of measures of treatment satisfaction and goal achievement. We believe that if a new multidimensional measure can be developed, then standardization of response definitions would allow for cross-trial comparisons and remove the confusion caused by individual symptom reporting while collecting data that are meaningful to both patients and practitioners.

2. Evidence acquisition

We conducted a narrative review of OAB literature available in the PubMed database. If an article that satisfied the study inclusion criteria was identified, two members of the research team (Kopp and Evans) reviewed the article's abstract for inclusion. If the two authors agreed, the full-text article was retrieved for analysis. A full-text article was excluded if its focus was not related to OAB outcome measures. The two researchers had to agree before an article

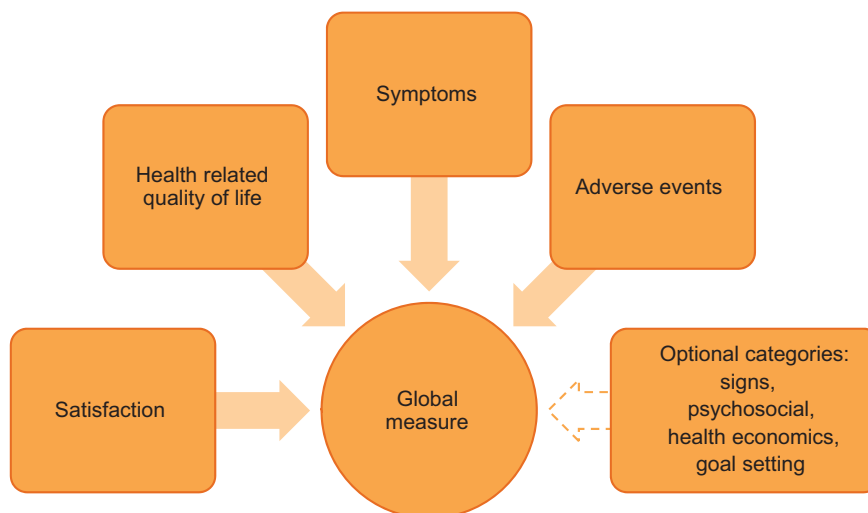


Fig. 1 – Recommended endpoints in overactive bladder.

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